

## Development of Methods for Identifying Child and Parent Health Outcomes

Bevans, Katherine

Children's Hospital of Philadelphia (CHOP)

The views of children regarding their health and treatment preferences are often devalued because of the erroneous assumption that they are unable to provide insight into their own health experiences. In fact, our research group and many others have demonstrated that children as young as age 8 can provide reliable, valid, and useful information about their health and outcome preferences. The question is no longer whether to incorporate the views of children into research that can affect their treatment decision-making, but how to do so. We intend to address this methodological gap by developing and evaluating novel methods for eliciting and prioritizing children's health outcomes that are comprehensible to both patients and clinicians, measurable, and ready for integration in clinical and CE research. Our specific aims are: (1) to develop a typology of health outcomes that is relevant to the experiences and preferences of children; (2) to ensure the comprehensiveness and comprehensibility of the health outcome typology for children and their parents; and, (3) to develop and test the feasibility and practicality of methods that use the typology to prioritize health outcomes from the perspectives of children and their families, using Autism Spectrum Disorder (ASD) as a proof-of-principle condition. We focus on ASD because of the broad range of anticipated outcome priorities, the strength of our partnership with advocates for this condition, and its inclusion in the IOM Top 100 CE priorities. The primary deliverables of this work are the pediatric health outcome typology and the evaluation of alternative approaches for eliciting and prioritizing health outcomes from the perspectives of children and their families. Through these innovations, we will begin to standardize methods used to integrate patients' perspectives into CE and clinical research. This project leverages our methodological expertise in understanding and accounting for the unique aspects of health in childhood. Our team is composed of international leaders in the patient reported outcome movement for children and families, the World Health Organization's efforts to classify health outcomes, parent advocacy, psychometrics, and pediatric healthcare. The long-term goals of this work are to extend the outcome typology to other conditions and age groups and catalyze the inclusion of core sets of patient-prioritized health outcomes into CE and clinical research to facilitate comparisons across studies and to engage patients as partners in the research enterprise.

### RELEVANCE

The Patient-Centered Outcome Research Institute (PCORI) aspires to improve the end-results of healthcare by advancing evidence-based medicine and ensuring that patients, caregivers, clinicians, and other stakeholders are engaged as full partners in the research enterprise. Although progress has been made in advancing methods for acquiring and synthesizing scientific evidence, there has been less attention given to integration of patients into research studies. Failure to incorporate patients' views in clinical and comparative effectiveness research may lead to the identification of 'best practice' interventions that have no or little effect on the outcomes that matter most to patients. This is especially true for children whose opinions about their health and treatment priorities tend to be ignored because of the fallacious assumption that they do not have the cognitive capabilities to engage in these discussions. The goal of the proposed pilot project is to develop and evaluate methods for eliciting and prioritizing health outcomes from the perspectives of children and their families. The key PCORI areas of interest that will be addressed by the proposed project are: (1) the development and testing of methods for eliciting and prioritizing outcomes from the perspectives of patients; (2) use of technology to assist with the outcome elicitation and prioritization processes; and, (3) the project's focus on children (a priority population). Our long-term goal of catalyzing the incorporation of core sets of patient-prioritized health outcomes into CE and clinical research is fully consonant with the mission of PCORI.